Blue Cross and Blue Shield of Kansas City (Blue KC) will provide coverage for a vertical expandable prosthetic titanium rib when it is determined to be medically necessary because the criteria shown below are met.

**When Policy Topic is covered**
Use of the vertical expandable prosthetic titanium rib is considered *medically necessary* in the treatment of progressive thoracic insufficiency syndrome due to rib and/or chest wall defects in infants/children between six months of age and skeletal maturity.

**When Policy Topic is not covered**
Use of the vertical expandable prosthetic titanium rib for all other conditions, including but not limited to the treatment of scoliosis in patients without thoracic insufficiency, is considered *investigational*.

**Considerations**
Skeletal maturity occurs at about age 14 for girls and age 16 for boys.

Given the complexity of these procedures and patients, implantation of this device should be performed in specialized centers. Preoperative evaluation requires input from pediatric orthopaedists, pulmonologist, and thoracic surgeon. In addition, preoperative evaluation of nutritional, cardiac and pulmonary function (when possible) is required.

**Description of Procedure or Service**

<table>
<thead>
<tr>
<th>Populations</th>
<th>Interventions</th>
<th>Comparators</th>
<th>Outcomes</th>
</tr>
</thead>
<tbody>
<tr>
<td>Individuals:</td>
<td>Interventions of interest are:</td>
<td>Comparators of interest are:</td>
<td>Relevant outcomes include:</td>
</tr>
<tr>
<td>▪ With progressive</td>
<td>▪ Vertical expandable prosthetic titanium rib</td>
<td>▪ Respiratory supportive care</td>
<td>▪ Symptoms</td>
</tr>
<tr>
<td>thoracic insufficiency syndrome due to rib and/or chest wall defects in childhood</td>
<td>thoracoplasty</td>
<td></td>
<td>▪ Morbid events</td>
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<td></td>
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<td>▪ Functional outcomes</td>
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<td>▪ Treatment-related mortality</td>
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<td></td>
<td></td>
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<td>▪ Treatment-related morbidity</td>
</tr>
<tr>
<td>Individuals:</td>
<td>Interventions of interest are:</td>
<td>Comparators of interest are:</td>
<td>Relevant outcomes include:</td>
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<tr>
<td>With early-onset scoliosis without thoracic insufficiency syndrome</td>
<td>Vertical expandable prosthetic titanium rib thoracoplasty</td>
<td>Bracing</td>
<td>Symptoms</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Spinal fusion</td>
<td>Morbid events</td>
</tr>
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</table>

The vertical expandable prosthetic titanium rib (VEPTR) is a curved rod placed vertically in the chest to help shape the thoracic cavity. It is being evaluated in skeletally immature patients with thoracic insufficiency syndrome (TIS) to support thorax and lung development and in pediatric patients with scoliosis without TIS to slow or correct curve progression.

For individuals who have progressive TIS who receive the VEPTR as a means of thoracoplasty, the evidence includes few case series. Relevant outcomes are symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. TIS occurs in a limited patient population. For example, the Boston Center reported results on 31 children treated from 1999 to 2005. The natural history of progressive TIS is worsening pulmonary function and pulmonary insufficiency. Results from case series reported at different specialty centers have demonstrated improvement and/or stabilization in key measures with use of the VEPTR in progressive TIS. This improvement has been noted in measures related to thoracic structure (eg, Cobb angle for those with scoliosis), growth of the thoracic spine and lung volumes, and stable or improved ventilatory status. While pulmonary function testing is difficult to track in patients suffering with TIS, one study managed to demonstrate an age-specific increase in forced vital capacity; further still, that same study was able to report a final forced vital capacity in the range of 50% to 70% of predicted value. Given the usual disease course of worsening thoracic volume and ventilatory status, the stabilization and/or improvement in these measures would be highly unlikely if not for the intervention. Taken together, these outcome measures demonstrate the positive impact of utilizing the VEPTR technology. The evidence is sufficient to determine that the technology results in a meaningful improvement in the net health outcome.

For individuals with early-onset scoliosis without TIS who receive the VEPTR as a means of thoracoplasty, the evidence includes few case series. Relevant outcomes are symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. The VEPTR is being evaluated for curves greater than 45° in infants and juveniles without thoracic insufficiency. Similar to TIS, very limited data are available on the use of the VEPTR for early-onset scoliosis without thoracic insufficiency; additionally, little is known about the disease progression of early-onset scoliosis, and therefore little is known regarding the risk-benefit tradeoff of the VEPTR® surgery. The evidence is insufficient to determine the effects of the technology on health outcomes.
Background
Thoracic insufficiency syndrome (TIS) is the inability of the thorax to support normal respiration or lung growth. (1) The condition results from serious defects affecting the ribs or chest wall, such as severe scoliosis, rib fusion (which may accompany scoliosis), and various hypoplastic thorax syndromes, such as Jeune syndrome and Jarcho-Levin syndrome. Spine, chest, and lung growth are interdependent (2). While the coexistence of chest wall and spinal deformity is well-documented, this effect on lung growth is not completely understood.

Progressive TIS includes respiratory insufficiency, loss of chest wall mobility, worsening 3-dimensional thoracic deformity, and/or worsening pulmonary function tests. As a child grows, progressive thoracic deformity and rotation toward the concave side occurs with worsening respiratory compromise. This progression is often accompanied by a need for supplemental oxygen and can require mechanical ventilation. While spinal fusion is one approach to treatment, it may not be successful and also may limit growth (lengthening) of the spine.

The vertical expandable prosthetic titanium rib (VEPTR) is a curved rod placed vertically in the chest that helps to shape the thoracic cavity. It is positioned either between ribs or between the ribs and either the spine or pelvis. The device is designed to be expanded every 4 to 6 months as growth occurs and also to be replaced if necessary. Some patients require multiple devices.

Regulatory Status
A VEPTR initially received approval from FDA under a humanitarian device exemption for the treatment of TIS in skeletally immature patients. TIS is defined as the inability of the thorax to support normal respiration or lung growth. In 2014, FDA cleared the VEPTR through the 510(k) process. The VEPTR/VEPTR II device is indicated for skeletally immature patients with severe progressive spinal deformities and/or 3-dimensional deformity of the thorax associated with or at risk of TIS. This would include patients with progressive congenital, neuromuscular, idiopathic, or syndromic scoliosis.

For the purpose of identifying potential TIS patients, the categories in which TIS patients fall are as follows:
- Flail chest syndrome
- Rib fusion and scoliosis
- Hypoplastic thorax syndrome, including,
  - Jeune's syndrome
  - Achondroplasia
  - Jarcho-Levin syndrome
  - Ellis van Creveld syndrome

Rationale
This evidence was created in April 2007 and has been updated regularly using the MEDLINE database. The most recent literature review was performed through June 22, 2017.
Thoracic insufficiency occurs in a limited patient population, and the literature on the use of the vertical expandable prosthetic titanium rib (VEPTR) consists mostly of case series from single institutions (some series are from specialized pediatric centers); no comparative trials have been identified. The following is a summary of the literature to date.

**THORACIC INSUFFICIENCY SYNDROME**

Data submitted to the U.S. Food and Drug Administration (FDA) on thoracic insufficiency syndrome (TIS) include an initial feasibility study involving 33 patients and a subsequent prospective study of 224 patients (214 with baseline data) at 7 study sites. Of these, 94 had rib fusion, 93 had hypoplastic thoracic syndrome, 46 had progressive scoliosis, and 14 had flail chest as a cause of their TIS. Three- and five-year follow-up rates for the multicenter study were approximately 95%. Of the 247 patients enrolled in either study, 12 (4.8%) patients died, and 2 withdrew. None of the deaths, as determined by investigators, were related to the VEPTR. Because standard pulmonary function testing was not possible for most of this population, an assisted ventilatory rating (AVR) was used to assess impact on respiratory status. The AVR ranged from 0 (unassisted breathing on room air) to 4 (full-time ventilatory support). In the multicenter prospective study, the AVR outcome improved or stabilized for 93% of the patients. Data were not reported for the number of patients who were no longer dependent on a ventilator.

Campbell (2004), who developed the VEPTR, and colleagues reported on 27 patients who had surgery for TIS and at least 2 years of follow-up data; this series was based on 41 patients treated between 1990 and the study reporting. Entry criteria for this study were acceptance by pediatric general surgeon, pediatric pulmonologist, and a pediatric orthopedist; age 6 months to skeletal maturity; progressive TIS; more than 10% reduction in height of the concave hemithorax; and 3 or more anomalous vertebrae, with 3 or more fused ribs at the apex of the deformity. Patients were followed for an average of 3.2 (range, 2-12) years. Before surgery, the mean annual rate of progression was $15^\circ$ per year (range, 2-50 years). Following surgery, the Cobb angle (of scoliosis) improved from $74^\circ$ to a final value of $49^\circ$. Spine growth was at a rate of 0.8 cm per year. (Normal spinal growth is 0.6 cm/year for ages 5-10 years.) The final forced vital capacity (FVC) was 49% of predicted value in the 19 children who could complete pulmonary function tests. Preoperatively, one patient required continuous positive airway pressure, and one needed supplemental oxygen for ventilatory support at final follow-up. Another publication from this group reported average 40.7-month follow-up (range, 25-78 months) in 24 children with nonsyndromic congenital scoliosis. Twenty-three (95.8%) children had associated rib fusions, and the average age at surgery was 3.3 years (range, 0.7-12.5 years). With a mean of 5 expansion surgeries per patient (range, 1-10), the Cobb angle improved by a mean of $8.9^\circ$ and thoracic height improved by a mean of 3.41 cm. Eight (33%) patients had a total of 16 adverse events, all of which required surgery.
In another series, Gadepalli et al (2011) examined growth and pulmonary function in 26 children who received a VEPTR between 2006 and 2010. The children underwent 29 insertions and 57 expansions, with an average of 3 surgeries per child. Each procedure required an average 0.97 days in the intensive care unit and 4.41 days in the hospital. The mean Cobb angle improved by 29%, from 64.7° preoperatively to 46.1° postoperatively. Lung volumes measured by yearly thoracic computed tomography (CT) scans were similar when corrected for age. Pulmonary function tests were performed every 6 months in patients (n=12) who were not ventilator-dependent and could cooperate with the procedure. Pulmonary function tests showed no significant change from baseline to follow-up in percent predicted values for forced expiratory volume in 1 second (54.6 vs 51.8), FVC (58.1 vs 55.9), or residual volume (145.3 vs 105.6), all respectively. Reoperation was required for 14 complications, 4 for chest tube placement (pneumothorax), 1 for seroma drainage, 6 for hardware removal (for infection), and 3 for hardware repositioning (for dislodgement). Another 22 complications were treated nonoperatively.

Emans et al (2005) reported results on patients with TIS who underwent the procedure at a single children’s hospital from 1999 to 2005. Thirty-one patients with fused ribs and TIS were treated; four patients had prior spinal arthrodesis with continued progression of deformity. Before surgery, all patients showed progressive spinal deformity, progressive chest deformity, or progressive hemithoracic constriction. The mean age was 4.2 years, and mean follow-up was 2.6 years (range, 0.5-5.4 years). A three-member team selected patients for surgery, and cardiac function was evaluated preoperatively. Surgery was performed using the Campbell technique for VEPTR. Lengthening of the VEPTR was planned for every four-to-six months but often was longer due to intercurrent illness or difficulty with travel. The mean number of device lengthenings was 3.5 (range, 0-10). Six patients had device exchanges for growth. In 30 patients, spinal deformity was controlled, and growth continued (1.2 cm/y) in the thoracic spine during treatment at rates similar to normal children. In this study, final FVC was 73.5% of predicted levels. Prior to the procedure, two patients were on ventilators and three patients required oxygen; at final follow-up, one patient required oxygen. Lung volume (measured by CT scan) in the operated lung increased from 157 cm³ preoperatively to 326 cm³ at the final follow-up visit.

Motoyama et al (2006) from a children’s hospital reported on 10 patients with TIS. Using a special portable PFT device, they reported on lung function in 10 children who had a VEPTR. Median age was 4.3 years (range, 1.8-9.8 years) at first test, and patients were followed an average of 22 months (range, 7-33 months). At baseline, FVC showed a moderate-to-severe decrease (69% of predicted), indicating the presence of significant restrictive lung defect. FVC increased significantly over time, with an average rate of 26.8% per year, similar to that of healthy children of comparative ages. In terms of percent predicted values, FVC did not change significantly between the baseline and last test (70.3%), indicating that, in most children studied, lung growth kept pace with body growth.
A series of 22 patients from another children’s hospital was published in 2007. (9) There is a number of additional series. Some have discussed weight gain after use of VEPTR in TIS (10) while others have discussed early changes in pulmonary function. (11)

**SCOLIOSIS WITHOUT THORACIC INSUFFICIENCY SYNDROME**

In 2011, White et al reported on the off-label use of spine-to-spine VEPTR to treat spinal deformity in 14 children without chest wall abnormalities. (12) The indications for the dual spine-to-spine rods were absence of a primary chest wall deformity, progression of spinal deformity to a Cobb angle of greater than 50°, and migration of a previously placed proximal rib anchor or a prior non-VEPTR growing rod to the point of loss of stable fixation. At final follow-up (24-48 months), there was an improvement in the Cobb angle from 74° to 57°, an increase in T1-S1 height from 260 to 296 mm, and no significant change in kyphosis. Complications occurred in 6 (43%) of 14 patients and included 3 rod fractures in 2 patients, 3 superficial infections, and 1 case of prominent hardware that threatened skin integrity. As noted by authors, while results were similar to those obtained with other growing rods, “the high complication rates, need for multiple procedures in growing children, and small relative gains in radiographic parameters still challenge proof of the efficacy of all such treatment methods.”

In 2014, treatment of congenital scoliosis with VEPTR (n=22) was compared with treatment with spinal fusion (n=27) and observation (n=184) based on a prospective registry. (13) Function, pain, and mental health status were measured with the 22-item Scoliosis Research Society questionnaire. Compared with the observation group, the VEPTR group had higher total and image scores at the second and third visits and higher function scores at the third and fourth visits. Interpretation of this study is limited due to confounding factors, including age at treatment, unknown comorbidities, and the rationale for treatment selection.

**ADVERSE EVENTS**

Complications that occur with VEPTR need to be considered by practitioners and families when discussing this procedure. Information on complications is summarized using data from the FDA review and the articles by Campbell and Emans. (3, 4, 7) Up to 25% of patients may experience device migration, including rib erosion. Approximately 10% of patients had infection-related complications. Brachial plexus injury or thoracic outlet syndrome occurred in 1% to 7% of these series. Skin sloughing was reported in 4 (15%) patients in the study by Campbell. In 1 single-center series reporting on complications for 65 patients treated for TIS over a 13-year period, device-related complications occurred in 22 patients. (14)

**SUMMARY OF EVIDENCE**

For individuals who have progressive TIS who receive the VEPTR as a means of thoracoplasty, the evidence includes few case series; relevant outcomes are: symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. TIS occurs in a limited patient population. For example, the Boston Center reported results on 31 children treated from 1999 to 2005. The natural
history of progressive TIS is worsening pulmonary function and pulmonary insufficiency. Results from case series reported at different specialty centers have demonstrated improvement and/or stabilization in key measures with use of the VEPTR® in progressive TIS. This improvement has been noted in measures related to thoracic structure (e.g., Cobb angle for those with scoliosis), growth of the thoracic spine and lung volumes, and stable or improved ventilatory status. While pulmonary function testing is difficult to track in patients suffering with TIS, one study managed to demonstrate an age-specific increase in forced vital capacity; further still, that same study was able to report a final forced vital capacity in the range of 50% to 70% of predicted value. Given the usual disease course of worsening thoracic volume and ventilatory status, the stabilization and/or improvement in these measures would be highly unlikely if not for the intervention. Taken together, these outcome measures demonstrate the positive impact of utilizing the VEPTR® technology. The evidence is sufficient to determine that the technology results in a meaningful improvement in the net health outcome.

For individuals with early-onset scoliosis without TIS who receive the VEPTR® as a means of thoracoplasty, the evidence includes few case series; relevant outcomes are: symptoms, morbid events, functional outcomes, and treatment-related mortality and morbidity. The VEPTR® is being evaluated for curves greater than 45° in infants and juveniles without thoracic insufficiency. Similar to TIS, very limited data are available on the use of the VEPTR® for early-onset scoliosis without thoracic insufficiency; additionally, little is known about the disease progression of early-onset scoliosis, and therefore little is known regarding the risk-benefit tradeoff of the VEPTR® surgery. The evidence is insufficient to determine the effects of the technology on health outcomes.

SUPPLEMENTAL INFORMATION

PRACTICE GUIDELINES AND POSITION STATEMENTS
No guidelines or statements were identified.

U.S. PREVENTIVE SERVICES TASK FORCE RECOMMENDATIONS
Not applicable.

MEDICARE NATIONAL COVERAGE
There is no national coverage determination. In the absence of a national coverage determination, coverage decisions are left to the discretion of local Medicare carriers.

ONGOING AND UNPUBLISHED CLINICAL TRIALS
Some currently unpublished trials that might influence this review are listed in Table 1.

Table 1. Summary of Key Trials

<table>
<thead>
<tr>
<th>NCT No.</th>
<th>Trial Name</th>
<th>Planned Enrollment</th>
<th>Completion Date</th>
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Unpublished

| NCT00689533a | VEPTR Implantation to Treat Children With Early Onset Scoliosis Without Rib Abnormalities: A Prospective Multicenter Study | 250 | Jan 2016 (unknown) |

NCT: national clinical trial.

a Denotes industry-sponsored or cosponsored trial.

References


Billing Coding/Physician Documentation Information

There is no specific code for this procedure. The procedure would most likely be reported with the unlisted code 22899.
**ICD-10 Codes**

- **M41.00-** Scoliosis code range
- **M41.9**
- **Q76.3** Congenital scoliosis due to congenital bony malformation
- **Q76.6** Other congenital malformations of ribs (includes congenital absence of rib and congenital fusion of ribs)
- **Q77.2** Osteochondrodysplasia with defects of growth of tubular bones and spine; short rib syndrome (includes Asphyxiating thoracic dysplasia [Jeune])
- **Q87.2** Congenital malformation syndromes predominantly involving limbs (includes VATER syndrome)

**Additional Policy Key Words**

N/A

**Policy Implementation/Update Information**

- 9/1/07 New policy.
- 9/1/08 No policy statement changes.
- 9/1/09 No policy statement changes.
- 9/1/10 No policy statement changes.
- 9/1/11 No policy statement changes.
- 9/1/12 No policy statement changes.
- 10/1/12 Material added on treatment of scoliosis without thoracic insufficiency (considered investigational)
- 10/1/13 No policy statement changes.
- 10/1/14 No policy statement changes.
- 10/1/15 No policy statement changes.
- 10/1/16 No policy statement changes.
- 10/1/17 No policy statement changes.

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State and Federal mandates and health plan contract language, including specific provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage. The medical policies contained herein are for informational purposes. The medical policies do not constitute medical advice or medical care. Treating health care providers are independent contractors and are neither employees nor agents Blue KC and are solely responsible for diagnosis, treatment and medical advice. No part of this publication may be reproduced, stored in a retrieval system or transmitted, in any form or by any means, electronic, photocopying, or otherwise, without permission from Blue KC.